BC PharmaCare Drug Information

The drug below is being considered for coverage under the BC PharmaCare program. PharmaCare is a government-funded drug plan that helps B.C. residents with the cost of eligible prescription drugs and medical supplies. For more information about PharmaCare, visit the PharmaCare website.

PharmaCare reviews each drug for treating a specific illness or medical condition (also called an "indication"). If PharmaCare decides to cover a drug, that coverage applies only to the indication(s) specified. In some cases, PharmaCare covers a drug only for people who have not responded to other drugs that treat the same indication.

More information about the PharmaCare drug review process is provided on the last page of this document.

Drug information	
Generic name (scientific name)	**trofinetide**
Brand name	**Daybue™**
Manufacturer	Acadia Pharmaceuticals Canada Inc
Indication	For the treatment of Rett syndrome (RTT) in adults and pediatric patients 2 years of age and older and weighing at least 9 kilograms.
Has the drug been reviewed by CDA-AMC, or will CDA-AMC be reviewing it? (See note below.)	Yes For more information about the CDA-AMC Reimbursement Review (CRR) of trofinetide (Daybue™), Search the CDA-AMC Reports.
Public input start date	**Wednesday,** February 26, 2025
Public input closing date	**Tuesday,** March 25, 2025, at **11:59 PM**
How is the drug taken?	Trofinetide is taken orally (through the mouth) or with a gastronomy (feeding) tube.
How often is the drug taken?	Trofinetide is taken twice a day, once in the morning and evening.

Drug information	
General drug and/or drug study information	Rett syndrome (RTT) is a rare, neurodevelopmental disorder that involves a progressive loss of speech, purposeful hand use, and motor skill. Common symptoms include stereotypic hand movements (such as wringing, clapping, or mouthing), gait abnormalities, breathing irregularities (like hyperventilation or breath-holding), seizures, and intellectual disabilities. RTT is most often caused by genetic variants in the MECP2 gene located on the X (female) chromosome, though it can occur in males in some cases. The MECP2 gene gives instructions for making a protein that is important for brain function and regulating the activity of other genes in the brain during development. It acts like a switch, turning genes on and off at the right time to help brain cells grow, communicate, and function properly. Trofinetide helps manage RTT by targeting the underlying cause of the disorder. However, the mechanism by which trofinetide offers therapeutic effects in patients with RTT is unknown. Studies looked at the following: • Changes in neurobehavioral symptoms, as measured by a caregiver-completed assessment, the RTT Behaviour Questionnaire (RSBQ) • Improvement or worsening compared to baseline, as measured by the Clinical Global Impression – Improvement (CGI-I) score • Communication through nonverbal means and symbolic behaviours, as measured by the RTT Clinician Rating of Ability to Communicate Choices (RTT-COMC) and the Communication and Symbolic Behaviour Scales Development Profile Infant-Toddler (CSBS-DP-IT) Checklist • Burden of caring for the patient on the daily life of the caregiver, as measured by the RTT Caregiver Burden Inventory (RTT-CBI) • The RTT Clinician Rating of Hand Function (RTT-HF), RTT Clinician Rating of Verbal Communication (RTT-VCOM), RTT Clinician Rating of Ambulation and Gross Motor Skills (RTT-AMB) • Bad reactions • Serious bad reactions • Patients leaving the trial due to bad reactions • Bad reactions of special interest, including vomiting and diarrhea

Drug information	
Other considerations	None

Note:
CDA-AMC (Canada's Drug Agency-L'Agence des Médicaments du Canada) is a national organization that reviews drugs on behalf of Canadian public sector plans when drug manufacturers want those plans to provide coverage for the drug. For detailed information about the PharmaCare drug review process, including the role of the CDA-AMC Reimbursement Review (CRR) in that process, visit How PharmaCare Decides Which Drugs to Cover.

Cost of the drug compared to other drugs used to treat the same indication				
Generic Name (Brand Name) of Drug Comparator	**PharmaCare Status (if and how the drug is already covered)**	**Dosage Form**	**Usual Dose**	**Annual Cost of Therapy[a]**
Trofinetide (Daybue)	Under Review	Oral solution for oral or gastronomy tube use	Twice daily doses by patient weight	$445,251[b] to $1,335,754[b]

[a] All prices as per PharmaCare formulary, unless otherwise specified. Weight-based dosing assumes a weight of 65 kg; dosing based on body surface area assumes an area of 1.8 m^2.
[b] Price as per CDA Pharmacoeconomic Review Report for trofinetide (Daybue).

The Drug Review Process in B.C.

A manufacturer submits a request to the Ministry of Health (Ministry).

An independent group called the Drug Benefit Council (DBC) gives advice to the Ministry. The DBC looks at:

- whether the drug is safe and effective
- advice from a national group called Canada's Drug Agency-L'Agence des médicaments du Canada (CDA-AMC)
- what the drug costs and whether it is a good value to the citizens of B.C.
- ethical considerations related to covering or not covering the drug
- input from physicians, patients, caregivers, patient groups and drug submission sponsors

The Ministry makes PharmaCare coverage decisions by taking into account:

- existing PharmaCare policies, programs and resources
- the evidence-informed advice of the DBC
- the drugs already covered by PharmaCare to treat similar medical conditions
- the overall cost of covering the drug

For more information about the drug review process in B.C., visit: the How PharmaCare decides which drugs to cover.

This document provides information only.
It does not take the place of advice from a physician or other qualified health care provider.